Pwyllgor Gwasanaethau Iechyd Arbenigol Cymru (PGIAC)
Welsh Health Specialised Services Committee (WHSSC)

Specialised Services
Policy Position Statement PP267

Selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over

September 2023

Version 1.0

PARCH - RESPECT
PARTNERIAETH - PARTNERSHIP
GWELLA AC ARLOESI - IMPROVEMENT & INNOVATION

Document information	
Document purpose	For information and action
Publication date	September 2023
Commissioning Team	Neurosciences and Long-term Conditions
Target audience	**For information** Chief Executives, Medical Directors, Directors of Finance, Directors of Planning, All Wales Medical Genetic Service
	For action Chief Pharmacists, Clinical Leads, Nurse Specialists, Paediatric Neurologists
Description	NHS Wales routinely commission this specialised service in accordance with the criteria described in this policy
Document No	PP267
Review Date	2026

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Policy Statement

Welsh Health Specialised Services Committee (WHSSC) will commission selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over in accordance with the criteria outlined in this document.

Welsh Language

WHSSC is committed to treating the English and Welsh languages on the basis of equality, and endeavour to ensure commissioned services meet the requirements of the legislative framework for Welsh Language, including the Welsh Language Act (1993), the Welsh Language (Wales) Measure 2011 and the Welsh Language Standards (No.7) Regulations 2018.

Where a service is provided in a private facility or in a hospital outside of Wales, the provisions of the Welsh language standards do not directly apply but in recognition of its importance to the patient experience, the referring health board should ensure that wherever possible patients have access to their preferred language.

In order to facilitate this, WHSSC is committed to working closely with providers to ensure that in the absence of a Welsh speaker, written information will be offered and people have access to either a translator or 'Language-line' if requested. Where possible, links to local teams should be maintained during the period of care.

Decarbonisation

WHSSC is committed to taking assertive action to reducing the carbon footprint through mindful commissioning activities. Where possible and taking into account each individual patient's needs, services are provided closer to home, including via digital and virtual access, with a delivery chain for service provision and associated capital that reflects the WHSSC commitment.

Disclaimer

WHSSC assumes that healthcare professionals will use their clinical judgment, knowledge and expertise when deciding whether it is appropriate to apply this policy position statement.

This policy may not be clinically appropriate for use in all situations and does not override the responsibility of healthcare professionals to make decisions appropriate to the circumstances of the individual patient, in consultation with the patient and/or their carer or guardian, or Local Authority.

WHSSC disclaims any responsibility for damages arising out of the use or non-use of this policy position statement.

1. Introduction

This Policy Position Statement has been developed for the planning and delivery of selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over for people resident in Wales. This service will only be commissioned by the Welsh Specialised Services Committee (WHSSC) and applies to residents of all seven Health Boards in Wales.

In creating this document WHSSC has reviewed the relevant guidance issued by the National Institute of Health and Care Excellence (NICE)[1] and has concluded that selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over should be made available.

1.1 Background

Type 1 Neurofibromatosis

Type 1 neurofibromatosis (NF1) is a rare, complex and incurable disease in which symptoms arise in early childhood and are lifelong. It is caused by mutations in the NF1 tumour suppressor gene. Because the condition is genetic, it is possible for multiple members of the same family to be affected. Clinical symptoms associated with NF1 vary from person to person and commonly begin in early childhood and continue in adulthood. It can present with a wide range of symptoms and can affect multiple organ systems including the nervous system, skin, bones and eyes.

People with NF1 also have an increased risk of neurological comorbidities such as autism, attention deficit hyperactivity disorder and mental health disorders and an increased risk of certain forms of cancer. For most people with NF1, the clinical course of the disease is uncertain. This can be a source of anxiety for people with NF1 and their parents or carers.

NF1 affects male and female patients in equal numbers; it also affects all races and ethnic groups equally.

Plexiform neurofibromas

Around 25% of people with NF1 develop non-malignant peripheral nerve sheath tumours called Plexiform Neurofibromas (PN). PN can affect multiple body regions and reach extremely large sizes. Most PN associated with NF1 are symptomatic, and can cause pain, disfigurement and difficulties with physical functioning.

[1] Overview | Selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over | Guidance | NICE

Although growth of PN may slow during adolescence, there can be significant growth in PN in adult life for some people and there is a higher chance of malignancy in disease progression after 18 years of age.

Current treatment

The only existing treatment effective at reducing or removing PN tumours is surgery. As PN are large and invasive, they present many difficulties in terms of surgical resection, and as a result approximately half of all patients with NF1 PN are considered inoperable. PN that have not been completely removed may grow back, and even PN which have been completely resected may recur in paediatric patients.

If a PN is inoperable, people have best supportive care, including pain management, physiotherapy, psychological support and sometimes procedures such as a tracheostomy to alleviate severe airway morbidities.

Selumetinib (Koselugo®)

Selumetinib is in a class of medications called kinase inhibitors. It works by blocking the action of the abnormal protein that signals the tumours to grow. This helps to stop or slow tumour growth. It has a marketing authorisation in the UK for the 'treatment of symptomatic, inoperable plexiform neurofibromas in paediatric patients with neurofibromatosis type 1 aged 3 years and above'[2].

1.2 Equality Impact Assessment

The Equality Impact Assessment (EQIA) process has been developed to help promote fair and equal treatment in the delivery of health services. It aims to enable Welsh Health Specialised Services Committee to identify and eliminate detrimental treatment caused by the adverse impact of health service policies upon groups and individuals for reasons of race, gender re-assignment, disability, sex, sexual orientation, age, religion and belief, marriage and civil partnership, pregnancy and maternity and language (Welsh).

This policy has been subject to an Equality Impact Assessment in line with guidance contained in CPL-026[3].

The Assessment demonstrates the policy is robust and there is no potential for discrimination or adverse impact. All opportunities to promote equality have been taken.

[2] Koselugo 10 mg hard capsules - Summary of Product Characteristics (SmPC) - (emc) (medicines.org.uk)
[3] https://whssc.nhs.wales/publications/corporate-policies-and-procedures/corp-026-eqia-policy/

An EQIA was also carried out by NICE during the evaluation of selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over. For further details, please refer to the NICE website[4].

[4] https://www.nice.org.uk/guidance/hst20/history

2. Recommendations

The recommendations below represent the views of NICE, arrived at after careful consideration of the evidence available. Health professionals are expected to take into account the relevant NICE guidance[5], alongside the individual needs, preferences and values of the patient.

2.1 Inclusion Criteria

Selumetinib is recommended, within its marketing authorisation, for treating symptomatic and inoperable plexiform neurofibromas (PN) associated with type 1 neurofibromatosis (NF1) in children aged 3 and over, only if the company provides selumetinib according to the commercial arrangement[5].

2.2 Continuation of Treatment

Healthcare professionals are expected to review a patient's health at regular intervals to ensure they are demonstrating an improvement to their health due to the treatment being given.

There will be a need to decide when to discontinue the medication in non-responders. If no improvement to a patient's health has been recorded then clinical judgement on the continuation of treatment must be made by the treating healthcare professional.

2.3 Acceptance Criteria

The service outlined in this specification is for patients ordinarily resident in Wales, or otherwise the commissioning responsibility of the NHS in Wales. This excludes patients who whilst resident in Wales, are registered with a GP practice in England, but includes patients resident in England who are registered with a GP Practice in Wales.

2.4 Transition arrangements

Transition arrangements should be in line with Transition from children's to adults' services for young people using health or social care services NICE guidance NG43 and the Welsh Government Transition and Handover Guidance.

Transition involves a process of preparation for young people and their families for their transition to adulthood and their transition to adult services. This preparation should start from early adolescence 12-13 year olds. The exact timing of this will ideally be dependent on the wishes of the

[5] Overview | Selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis in children aged 3 and over | Guidance | NICE

young person but will need to comply with local resources and arrangements.

The transition process should be a flexible and collaborative process involving the young person and their family as appropriate and the service.

The manner in which this process is managed will vary on an individual case basis with multidisciplinary input often required and patient and family choice taken into account together with individual health board and environmental circumstances factored in.

2.5 Designated Providers

Selumetinib treatment will be initiated and monitored by the NF1 MDT at one of the following specialist UK centres

- Guy's and St Thomas' NHS Foundation Trust / Evelina London Children's Hospital.
- Manchester University NHS Foundation Trust / Royal Manchester Children's Hospital.

2.6 Blueteq and reimbursement

Selumetinib for treating symptomatic and inoperable plexiform neurofibromas associated with type 1 neurofibromatosis for children aged 3 and over will only be funded for patients registered via the Blueteq system and where an appropriately constructed MDT has approved its use.

Where the patient meets the criteria in this policy and the referral is received by an agreed centre, a Blueteq form should be completed for approval. For further information on accessing and completing the Blueteq form please contact WHSSC using the following e-mail address: WHSSC.blueteq@wales.nhs.uk

If a non-contracted provider wishes to treat a patient that meets the criteria they should contact WHSSC (e-mail: Wales.IPC@wales.nhs.uk). They will be asked to demonstrate they have an appropriate MDT in place.

Funding is approved on the basis that selumetinib is prescribed and administered in accordance with its marketing authorisation[6].

Selumetinib is available as a pack of 60 capsules. The cost per pack of 10mg capsules is £4,223.59 and per pack of 25mg capsules is £10,560 (excluding VAT) company submission. The company has a commercial arrangement. This makes selumetinib available to the NHS with a discount. The size of the discount is commercial in confidence. Health Boards in Wales

[6] Selumetinb - (emc) (medicines.org.uk)

should refer to the AWTTC Vault for further information on commercial arrangements.

2.7 Action to be taken

- Health Boards and WHSSC are to circulate this Policy Position Statement to all Hospitals/MDTs to inform them of the conditions under which the technology will be commissioned.
- WHSSC are to ensure that all providers are purchasing selumetinib at the agreed discounted price.
- Providers are to ensure the need to approve selumetinib at the appropriate MDT and are registering use on the Blueteq system, and the treatment will only be funded where the Blueteq minimum dataset is fully and accurately populated.
- Providers are to determine estimated patient numbers and the current dose of any patient(s) who will transfer from any company compassionate use scheme or EAMS.
- The Provider should work to written quality standards and provide monitoring information to WHSSC on request.

3. Putting Things Right

3.1 Raising a Concern

Whilst every effort has been made to ensure that decisions made under this policy are robust and appropriate for the patient group, it is acknowledged that there may be occasions when the patient or their representative are not happy with decisions made or the treatment provided.

The patient or their representative should be guided by the clinician, or the member of NHS staff with whom the concern is raised, to the appropriate arrangements for management of their concern.

If a patient or their representative is unhappy with the care provided during the treatment or the clinical decision to withdraw treatment provided under this policy, the patient and/or their representative should be guided to the LHB for NHS Putting Things Right. For services provided outside NHS Wales, the patient or their representative should be guided to the NHS Trust Concerns Procedure, with a copy of the concern being sent to WHSSC.

3.2 Individual Patient Funding Request (IPFR)

If the patient does not meet the criteria for treatment as outlined in this policy, an Individual Patient Funding Request (IPFR) can be submitted for consideration in line with the All Wales Policy: Making Decisions on Individual Patient Funding Requests. The All Wales IPFR Panel will then consider the request.

If an IPFR is declined by the Panel, a patient and/or their NHS clinician has the right to request information about how the decision was reached. If the patient and their NHS clinician feel the process has not been followed in accordance with this policy, arrangements can be made for an independent review of the process to be undertaken by the patient's Local Health Board. The ground for the review, which are detailed in the All Wales Policy: Making Decisions on Individual Patient Funding Requests (IPFR), must be clearly stated

If the patient wishes to be referred to a provider outside of the agreed pathway, an IPFR should be submitted.

Further information on making IPFR requests can be found at Welsh Health Specialised Services Committee (WHSSC) | Individual Patient Funding Requests